IND-enabling activities for a Phase 1 Study of Autologous CD4LVFOXP3 T Cells in Subjects with IPEX Syndrome

Grant Award Details

IND-enabling activities for a Phase 1 Study of Autologous CD4LVFOXP3 T Cells in Subjects with IPEX Syndrome

Grant Type: Late Stage Preclinical Projects

Grant Number: CLIN1-11591

Investigator:

Name:	Rosa Bacchetta
Institution:	Stanford University
Type:	PI

Disease Focus: Blood Disorders, Immune Disease, IPEX Syndrome

Award Value: $5,527,984

Status: Pre-Active

Grant Application Details

Application Title: IND-enabling activities for a Phase 1 Study of Autologous CD4LVFOXP3 T Cells in Subjects with IPEX Syndrome

Public Abstract:

Therapeutic Candidate or Device

CD4+ T cells that have undergone lentiviral -mediated gene transfer of Forkhead Box P3 (FOXP3) and acquired regulatory T cell function.

Indication

Immune dysregulation Polyendocrinopathy Enteropathy X-linked (IPEX) syndrome

Therapeutic Mechanism

Administration of autologous CD4LVFOXP3 that constitutively and stably express wild-type FOXP3 gene will replace the lack of functional regulatory T cells in patients with IPEX syndrome, a life-threatening pediatric disease due to FOXP3 gene mutation, and a prototype of genetic autoimmune disease.

Unmet Medical Need

IPEX has early severe onset and is a serious clinical challenge. Pharmacological immunosuppression can only partially control autoimmune manifestations and does not prevent organ damage. Allogeneic HSCT can cure but lack of suitable donors and transplant complications lead to inferior outcomes.

Project Objective

Filing of IND application with the FDA

Major Proposed Activities

- Complete nonclinical IND enabling safety and efficacy studies to meed the FDA requests.
- GMP FOXP3 lentiviral vector production to generate CD4LVFOXP3 cell product for clinical use and establish its GMP manufacturing process and scale up.
- File an Investigator New Drug (IND) application with the FDA to obtain approval to start the phase I clinical trial

Statement of Benefit to California:

IPEX syndrome not only impacts the affected patients but also directly impacts families and communities. Thus, improve treatment for IPEX patients would have tremendous personal benefit on the patients and their families and would provide a great economic benefit to the state. A successful outcome of this Treg replacement therapy in IPEX could support development of CD4LVFOXP3 cell product for other diseases with autoimmunity and immune dysregulation (e.g. IBD, T1D, scleroderma, acute GVHD).

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